



# Long Term Extension Study in Patients With Primary Hyperoxaluria

04/04/2025 15:26:47

## Main Information

**Primary registry identifying number**

LBCTR2020124677

**Protocol number**

DCR-PHXC-301

**MOH registration number**

NCT04042402

**Study registered at the country of origin**

No

**Study registered at the country of origin: Specify**

Study registered in clinicaltrials.gov

**Type of registration**

Prospective

**Type of registration: Justify**

N/A

**Date of registration in national regulatory agency**

02/08/2019

**Primary sponsor**

Dicerna Pharmaceuticals, Inc.

**Primary sponsor: Country of origin**

USA

**Date of registration in primary registry**

20/02/2021

**Date of registration in national regulatory agency**

02/08/2019

**Public title**

Long Term Extension Study in Patients With Primary Hyperoxaluria

**Acronym****Scientific title**

An Open-Label Roll-Over Study to Evaluate the Long-Term Safety and Efficacy of DCR-PHXC Solution for Injection (Subcutaneous Use) in Patients With Primary Hyperoxaluria

**Acronym**

"PHYOX3"

**Brief summary of the study: English**

The proposed study is designed to provide patients previously enrolled in Phase 1 and 2 studies of DCR-PHXC long-term access to DCR-PHXC, and to evaluate the long-term safety and efficacy of DCR-PHXC in patients with PH.

**Brief summary of the study: Arabic**

بالوصول طويل الأمد DCR-PHXC تم تصميم الدراسة المقترحة لتزويد المرضى المسجلين سابقاً في دراسات المرحلتين الأولى والثانية من PH على المدى الطويل في مرضى DCR-PHXC ولتقييم سلامة وفعالية ، DCR-PHXC إلى PH.

**Health conditions/problem studied: Specify**

Primary Hyperoxaluria Type 1 (PH1)  
Primary Hyperoxaluria Type 2 (PH2)  
Kidney Diseases  
Urologic Diseases  
Genetic Disease

**Interventions: Specify**

Drug: DCR-PHXC  
Multiple fixed doses of DCR-PHXC by subcutaneous (SC) injection.  
Other Name: Nedosiran

**Key inclusion and exclusion criteria: Inclusion criteria**

Key Inclusion Criteria:



•Participant successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC.

OR Participant is the sibling of a participant who successfully completed a Dicerna Pharmaceuticals, Inc. study of DCR PHXC. Siblings must be younger than 18 years of age and must have genetically confirmed PH.

•For participants rolling over from a multidose study of DCR-PHXC, enrollment should occur within a window of 25 to 60 days from the last dose of study intervention. Estimated GFR at screening  $\geq 30$  mL/min normalized to 1.73 m<sup>2</sup> body surface area (BSA), calculated using Chronic Kidney Disease Epidemiology Collaboration (CKD EPI) formula in participants aged  $\geq 18$  years (Levey & Stevens, 2010), or the formula by Schwartz in participants aged 6 to 16 years (Schwartz et al., 2009; National Kidney Foundation, 2002). In Japan, the formula by Uemura et al. will be used for participants aged 6 to 17 years (Uemura et al., 2014).

**Key inclusion and exclusion criteria: Gender**

Both

**Key inclusion and exclusion criteria: Specify gender**

**Key inclusion and exclusion criteria: Age minimum**

6

**Key inclusion and exclusion criteria: Age maximum**

99

**Key inclusion and exclusion criteria: Exclusion criteria**

Key exclusion criteria include:

- Renal or hepatic transplantation; prior or planned within the study period
- Current dialysis
- Documented evidence of clinical manifestations of systemic oxalosis (including pre-existing retinal, heart, or skin calcifications, or history of severe bone pain, pathological fractures, or bone deformations).

**Type of study**

Interventional

**Type of intervention**

Pharmaceutical

**Type of intervention: Specify type**

N/A

**Trial scope**

Other

**Trial scope: Specify scope**

**Study design: Allocation**

Single Arm Study

**Study design: Masking**

Open (masking not used)

**Study design: Control**

N/A

**Study phase**

3

**Study design: Purpose**

Treatment

**Study design: Specify purpose**

N/A

**Study design: Assignment**

Single

**Study design: Specify assignment**

N/A

**IMP has market authorization**

No

**IMP has market authorization: Specify**

**Name of IMP**

DCR PHXC

**Year of authorization**

**Month of authorization**

**Type of IMP**

Others

**Pharmaceutical class**

A synthetic double-stranded (hybridized duplex) ribonucleic acid (RNA) oligonucleotide conjugated to N-acetyl-D-galactosamine (GalNAc) amino-sugar residues.

**Therapeutic indication**

Primary Hyperoxaluria.



## Therapeutic benefit

At present, no therapies are approved by regulatory authorities for the treatment of patients with PH. DCR-PHXC treatment has the potential benefit to reduce or eliminate the excess oxalate production in the liver and thus avoid the need for a combined liver and kidney transplantation in patients not already on renal replacement therapy.

## Study model

N/A

## Study model: Explain model

N/A

## Study model: Specify model

N/A

## Time perspective

N/A

## Time perspective: Explain time perspective

N/A

## Time perspective: Specify perspective

N/A

## Target follow-up duration

## Target follow-up duration: Unit

## Number of groups/cohorts

## Biospecimen retention

None retained

## Biospecimen description

Blood and Urine Samples

## Target sample size

50

## Actual enrollment target size

## Date of first enrollment: Type

Anticipated

## Date of first enrollment: Date

01/02/2021

## Date of study closure: Type

Anticipated

## Date of study closure: Date

30/12/2023

## Recruitment status

Other

## Recruitment status: Specify

Enrolling by Invitation

## Date of completion

30/12/2023

## IPD sharing statement plan

No

## IPD sharing statement description

Participants will be assigned a unique identifier by the Sponsor. Any participant records or datasets that are transferred to the Sponsor will contain the identifier only; participant names or any information which would make the participant identifiable will not be transferred.



Additional data URL

Admin comments

Trial status

Approved

## Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
US NCT Number	NCT04042402

## Sources of Monetary or Material Support

Name
Dicerna pharmaceuticals inc. 75 Hayden Avenue Suite 400 Lexington, MA 02421, USA

## Secondary Sponsors

Name
NA

## Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Chadi Safa	lebanon. Baabda. Chiah. Ain el remeneh	Lebanon	009617125 1819	Chadi.safa@clin art.net	Clinart
Scientific	Chebl Mourani	Alfred Naccache Blvd, External Viewing Tower, Floor 4, Room 9403	Lebanon	03 290090	cheblmourani@g mail.com	HDF

## Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
1.Hotel Dieu de France	Chebl Mourani	Pediatric Nephrologist	Approved
2.Saint George University Hospital	Pauline Abu jaoude	Nephrologist	Approved



## Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	30/03/2020	Nancy Choukair Alam	nancy.alam@usj.edu.lb	: +961 1 421 000
Saint George Hospital University Medical Center	09/07/2020	Sandra Berberi	smberbari@stgeorgehospital.org	+961 1 44 16 30

## Countries of Recruitment

Name
France
Netherlands
Germany
United Kingdom
United States of America
Lebanon
Spain
Italy
Australia
Canada
Japan

## Health Conditions or Problems Studied

Condition	Code	Keyword
Primary Hyperoxaluria	2-Propanol (T51.2)	Kidney Diseases

## Interventions

Intervention	Description	Keyword
DCR-PHXC	Multiple fixed doses of DCR-PHXC by subcutaneous (SC) injection	Nedosiran



## Primary Outcomes

Name	Time Points	Measure
To evaluate the effect of DCR PHXC on estimated glomerular filtration rate	Annual change from baseline	estimated glomerular filtration rate

## Key Secondary Outcomes

Name	Time Points	Measure
The incidence and severity of treatment-emergent adverse events (TEAE) and SAEs associated with abnormal 12 lead electrocardiogram (ECG) readings	TEAEs and SAEs are evaluated monthly for 3 years	Electrocardiogram (ECG)

## Trial Results

**Summary results**

**Study results globally**

**Date of posting of results summaries**

**Date of first journal publication of results**

**Results URL link**

**Baseline characteristics**

**Participant flow**

**Adverse events**

**Outcome measures**

**URL to protocol files**